2018 Current Fiscal Year Report: Pediatrics Advisory Committee

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1. Department or Agency	2. Fiscal Year
Department of Health and Human Services	2018

3. Committee or Subcommittee	3b. GSA Committee No.
Pediatrics Advisory Committee	21515

4. Is this New During Fiscal Year?	5. Current Charter	6. Expected Renewal Date	7. Expected Term Date
No	09/27/2012		

8a. Was Terminated During FiscalYear?	8b. Specific Termination Authority	8c. Actual Term Date
No		

9. Agency Recommendation for Next FiscalYear	10a. Legislation Req to Terminate?	10b. Legislation Pending?
Continue	Not Applicable	Not Applicable

11. Establishment Authority Statutory (Congress Created)

12. Specific Establishment Authority	13. Effective Date	14. Commitee Type	14c. Presidential?
Public Law 107-109, Public Law 108-155, FDAAA	01/07/2003	Continuing	No

15. Description of Committee Scientific Technical Program Advisory Board

16a. Total Number of Reports No Reports for this FiscalYear

17a. Open 1 **17b. Closed** 0 **17c. Partially Closed** 1 **Other Activities** 0 **17d. Total** 2

Meetings and Dates

Purpose	Start	End
The Pediatric Advisory Committee and Endocrinologic and Metabolic Drug Advisory Committee met to discuss drug development for the treatment of children with achondroplasia. The following topics were discussed: Evidence required to establish dose-response, study design, study duration, intended population, and endpoints.	05/11/2018	05/11/2018
The Pediatric Advisory Committee met to discuss pediatric-focused safety reviews for INTUNIV and LEXAPRO. The Food and Drug Administration (FDA) will provide general safety updates including updates on the following topics without vote by the committee: • Overview of FDA's Adverse Event Reporting System and lack of efficacy; • Generic drug approval process; and discussion on trade versus generic drugs; exceptions; • Summary of FDA completed review of pediatric safety issues and updated labeling changes for Exjade (deferasirox); • Update labeling change for inhaled corticosteroid long-acting -2 agonists (ICS/LABAs); • Safety labeling for gadolinium.	09/20/2018	09/20/2018

Number of Committee Meetings Listed: 2

	Current FY	Next FY
18a(1). Personnel Pmts to Non-Federal Members	$19,906.00	$24,608.00
18a(2). Personnel Pmts to Federal Members	$0.00	$0.00
18a(3). Personnel Pmts to Federal Staff	$1,413,350.00	$1,426,406.00

18a(4). Personnel Pmts to Non-Member Consultants	$5,238.00	$8,203.00
18b(1). Travel and Per Diem to Non-Federal Members	$23,638.00	$29,665.00
18b(2). Travel and Per Diem to Federal Members	$0.00	$0.00
18b(3). Travel and Per Diem to Federal Staff	$0.00	$0.00
18b(4). Travel and Per Diem to Non-member Consultants	$8,618.00	$10,826.00
18c. Other(rents,user charges, graphics, printing, mail, etc.)	$392,055.00	$405,490.00
18d. Total	$1,862,805.00	$1,905,198.00
19. Federal Staff Support Years (FTE)	7.30	7.30

20a. How does the Committee accomplish its purpose?

The committee makes recommendations to the Commissioner of Food and Drugs in response to specific questions posed by the FDA. Recommendations for regulatory or policy decisions are reviewed by FDA staff and by the Commissioner, who then implements changes or forwards recommendations to the Department of Health and Human Services.

20b. How does the Committee balance its membership?

Members are authorities knowledgeable in pediatric research, pediatric subspecialties, statistics, and/or biomedical ethics. Members also include a patient-family representative , one technically qualified consumer representative, and may include one non-voting industry representative and one non-voting representative from a pediatric health organization.

20c. How frequent and relevant are the Committee Meetings?

The Pediatric Advisory Committee met two times in FY18 to cover critical regulatory and policy issues in the area of pediatric therapeutics which are essential for the agency to play a lead regulatory and policy role.

20d. Why can't the advice or information this committee provides be obtained elsewhere?

The Pediatric Advisory Committee is mandated by law. In addition, the committee will have advisory functions for many pediatric products regulated by the FDA. The committee will provide expert advice on specific regulatory and policy areas related to pediatric therapeutics, including (1) pediatric research conducted under sections 351, 409I, and 499 of the Public Health Service Act and sections 501, 502, 505, 505A, and 505B of the Federal Food, Drug, and Cosmetic Act; (2) identification of research priorities related to pediatric therapeutics and the need for additional treatments of specific pediatric diseases or conditions, (3) the ethics, design, and analysis of clinical trials related to pediatric

therapeutics, (4) pediatric labeling disputes as specified in section 3 of the Best Pharmaceuticals for Children Act (Public Law 107-109), (5) pediatric labeling changes as specified in section 5 of the Best Pharmaceuticals for Children Act (Public Law 107-109), (6) adverse event reports for drugs granted pediatric exclusivity and any safety issues that may occur as specified in section 17 of the Best Pharmaceuticals for Children Act (Public Law 107-109), (7) any other pediatric issue or pediatric labeling dispute involving FDA-regulated products, (8) research involving children as subjects as specified in 21 CFR 50.54, and (9) any other matter involving pediatrics for which FDA has regulatory responsibility. The Committee also advises and makes recommendations to the Secretary directly or to the Secretary through the Commissioner of Food and Drugs on research involving children as subjects that is conducted or supported by the Department of Health and Human Services as specified in 45 CFR 46.407.

20e. Why is it necessary to close and/or partially closed committee meetings?

FDA is required to partially close a portion of the meeting to permit discussion of trade secrets and/or commercial information 5 U.S.C. 522b(c)(4).

21. Remarks

There were no reports required for FY18. As provided in section 14(d) of the Best Pharmaceuticals for Children Act as amended by section 507 of the Food and Drug Administration Safety and Innovation Act of 2012, Pub. L. 112-144, notwithstanding section 14 of the Federal Advisory Committee Act, the Pediatric Advisory Committee will continue to operate to carry out the advisory committee’s responsibilities under sections 505A, 505B, and 520(m) of the Federal Food, Drug, and Cosmetic Act. During FY 2018, the Pediatric Advisory Committee was convened on 3 separate occasions for a total of 4 meeting days which included one day for training. The training day was added in order to educate the committee on matters pertaining to pediatric development and general administrative process. Accordingly, the Cost Section of this report reflects a total of 4 days of committee activity for FY 2018. On May 3, 2018, the Gastrointestinal Drugs Advisory Committee met jointly with the Pediatric Advisory Committee to discuss a new drug application for stannsoporfin injection, for intramuscular use, submitted by InfaCare Pharmaceutical Corporation, proposed for the treatment of neonates greater than or equal to 35 weeks of gestational age with indicators of hemolysis who are at risk of developing severe hyperbilirubinemia.

Designated Federal Officer

Marieann R Brill Associate Director for Regulatory Affairs, Office of Pediatric Therapeutics

Committee Members	Start	End	Occupation	Member Designation
Anne, Premchand	07/01/2018	06/30/2022	St. John Providence Children's Hospital	Special Government Employee (SGE) Member

Boyce, Danielle	01/03/2018	06/30/2021	Independent Consulting	Representative Member
Callahan, David	07/01/2017	06/30/2021	Washington University School of Medicine	Special Government Employee (SGE) Member
Cataletto, Mary	07/01/2014	06/30/2019	Winthrop University Hospital; Division of Pedaitric Pulmonology	Special Government Employee (SGE) Member
Cnaan, Avital	06/29/2014	06/30/2018	Children's National Medical Center; Division of Biostatistics and Study Methodology	Special Government Employee (SGE) Member
Cunningham, Melody	06/08/2016	06/30/2019	Le Bonheur Children's Hospital, Memphis, TN	Special Government Employee (SGE) Member
Dracker, Robert	06/30/2014	06/30/2019	Children's Hospital at Strong Memorial and Summerwood Pediatrics, Liverpool NY	Special Government Employee (SGE) Member
Flick, Randall	07/01/2018	06/30/2022	Mayo Clinic Children's Center	Special Government Employee (SGE) Member
Havens, Peter	06/06/2016	06/30/2020	Children's Hospital of Wisconsin, Milwaukee, WI	Special Government Employee (SGE) Member
Hoehn, K. Sarah	06/06/2016	06/30/2020	University of Kansas School of Medicine, Kansas, KS	Special Government Employee (SGE) Member
Hudak, Mark	02/13/2013	06/30/2018	University of Florida Health Science Center, Jacksonville, Fl	Special Government Employee (SGE) Member
Jones, Bridgette	01/23/2015	06/30/2019	Children's Mercy Hospital, Associate Professor of Pediatrics and Medicine	Representative Member
Moore, Erin	07/01/2016	10/29/2017	Cincinnati Children's Hospital Anderson Center for Health Systems Excellence	Representative Member
Oster, Randi	04/24/2018	06/30/2022	Help Me Health	Special Government Employee (SGE) Member
Portman, Ronald	03/19/2018	06/30/2022	Novartis	Representative Member
Turer, Christie	06/06/2016	06/30/2019	UT Southwestern and Children’s Medical Center, Dallas, TX	Special Government Employee (SGE) Member
Wade, Kelly	06/08/2016	06/30/2020	Pennsylvania Hospital, Philadelphia, PA	Special Government Employee (SGE) Member
Wael, Sayej	09/30/2016	06/30/2019	University of Connecticut School of Medicine	Special Government Employee (SGE) Member
White, Michael	07/01/2013	06/30/2018	Ochsner Clinic Foundation, New Orleans, LA	Special Government Employee (SGE) Member

Number of Committee Members Listed: 19

Narrative Description

FDA’s strategic priorities in responding to the public health challenges of the 21st century are to advance regulatory science and innovation; strengthen the safety and integrity of the global supply chain; strengthen compliance and enforcement activities to support public health; expand efforts to meet the needs of special populations; advance medical countermeasures and emergency preparedness; advance food safety and nutrition; promote public health by advancing the safety and effectiveness of medical products; establish an effective tobacco regulation, prevention, and control program; and manage for organizational excellence and accountability. The Pediatric Advisory Committee supports FDA’s strategic priorities by providing advice and making recommendations to the Commissioner of Food and Drugs on matters relating to pediatric therapeutics, pediatric research, and any other matter involving pediatrics for which the Food and Drug

Administration has regulatory responsibility. The Committee also advises and makes recommendations to the Secretary pursuant to 45 CFR 46.407 on research involving children as subjects that is conducted or supported by the Department of Health and Human Services. The recommendations of this committee support the agency by improving patient and consumer safety.

What are the most significant program outcomes associated with this committee?

	Checked if Applies
Improvements to health or safety	✓
Trust in government	✓
Major policy changes	✓
Advance in scientific research	✓
Effective grant making	
Improved service delivery	
Increased customer satisfaction	✓
Implementation of laws or regulatory requirements	✓
Other	

Outcome Comments

NA

What are the cost savings associated with this committee?

	Checked if Applies
None	
Unable to Determine	✓
Under $100,000	
$100,000 - $500,000	
$500,001 - $1,000,000	
$1,000,001 - $5,000,000	
$5,000,001 - $10,000,000	
Over $10,000,000	
Cost Savings Other	

Cost Savings Comments

The utilization of the Pediatric Advisory Committee (and subcommittees) enabled the Agency to obtain required and frequently scarce professional services from medical and scientific experts not otherwise available to the Agency; and to obtain the services of these experts only on an as needed bases rather than on a full time basis. The service of the committee resulated in advice for the improvement of the public health, for which it is

difficult to assign a financial value.

What is the approximate Number of recommendations produced by this committee for the life of the committee?
359

Number of Recommendations Comments
The committee made 359 recommendations from June of FY03 through FY18.

What is the approximate Percentage of these recommendations that have been or will be Fully implemented by the agency?
90%

% of Recommendations Fully Implemented Comments
The function of an advisory committee is purely advisory in nature. Although the FDA most often accepts the recommendations of its committees, the advice is purely advisory in nature, and therefore, the Agency has the option of not implementing the advice.

What is the approximate Percentage of these recommendations that have been or will be Partially implemented by the agency?
10%

% of Recommendations Partially Implemented Comments
The function of an advisory committee is purely advisory in nature. Although the FDA most often accepts the recommendations from its committees, the advice is purely advisory in nature, therefore, the Agency has the option of not implementing the advice.

Does the agency provide the committee with feedback regarding actions taken to implement recommendations or advice offered?
Yes ☑ No ☐ Not Applicable ☐

Agency Feedback Comments
When appropriate, information is made available to the public.

What other actions has the agency taken as a result of the committee's advice or recommendation?

	Checked if Applies
Reorganized Priorities	☑
Reallocated resources	☐

Issued new regulation	☑
Proposed legislation	☐
Approved grants or other payments	☐
Other	☑

Action Comments

The agency has made product labeling changes as a result of committee recommendations.

Is the Committee engaged in the review of applications for grants?

No

Grant Review Comments

NA

How is access provided to the information for the Committee's documentation?

	Checked if Applies
Contact DFO	☑
Online Agency Web Site	☑
Online Committee Web Site	☑
Online GSA FACA Web Site	☑
Publications	☑
Other	☐

Access Comments

N/A